



Ziopharm Oncology Presents Clinical Data Demonstrating Anti-tumor Immune Response of Controlled IL-12 Platform in Breast Cancer and Glioblastoma at American Society of Clinical Oncology (ASCO) Annual Meeting

June 4, 2018

- *Ad-RTS-hIL-12 plus veledimex recruits T cells into breast and brain cancers*
- *Median overall survival reaches 12.7 months in patients with recurrent glioblastoma in 20mg veledimex cohort who received Controlled IL-12 as a single agent*
- *Over expression of immune checkpoint biomarkers demonstrated*
- *Abscopal anti-tumor effect documented in metastatic breast cancer*
- *Data strongly support development of Controlled IL-12 platform in combination with immune checkpoint inhibitors*

BOSTON, June 04, 2018 (GLOBE NEWSWIRE) -- [Ziopharm Oncology](#), Inc. (Nasdaq:ZIOP), a biotechnology company focused on development of next generation immunotherapies utilizing gene- and cell-based therapies to treat patients with cancer, today presented clinical data showing the Company's Controlled IL-12 platform as monotherapy achieved anti-tumor responses in patients with metastatic breast cancer (mBC) and patients with recurrent glioblastoma (rGBM) at the [2018 American Society of Clinical Oncology \(ASCO\) Annual Meeting](#) in Chicago.

The poster, "Demonstration of Anti-Tumor Immunity via Intratumoral Regulated Platform Ad-RTS-hIL-12 in Advanced Breast Cancer and Recurrent Glioblastoma Patients," presented data from two open-label trials that evaluated Ad-RTS-hIL-12 plus veledimex, a gene therapy designed to induce and control the expression of the powerful cytokine interleukin 12 (IL-12). The poster is available on the [Company's website](#).

Updated data from the Company's Phase 1 rGBM study shows median overall survival (mOS) of 12.7 months has been sustained for patients treated with Ad-RTS-hIL-12 plus 20mg of veledimex (n=15) at a mean follow-up time of 12.9 months as of May 4, 2018. This mOS of 12.7 months continues to compare favorably to the 5 to 8 months survival established in historical controls for patients with rGBM.

"We remain excited that Ad-RTS-hIL-12 plus veledimex as monotherapy demonstrates promising antitumor responses and makes cold tumors hot with new immune infiltrating cells and overexpression of checkpoints," said Francois Lebel, M.D., Ziopharm's Chief Medical Officer and Executive Vice President for Research & Development. "We look forward to further development of our Controlled IL-12 platform in combination with immune checkpoint inhibitors, with one combination trial initiated in brain cancer and plans to advance a similar treatment regimen in a second tumor type later this year."

In both rGBM and mBC trials, an adenovirus vector with coding for the RheoSwitch Therapeutic System[®] (RTS[®]) genetic switch and IL-12 was injected intratumorally and patients took oral doses of veledimex, an activator ligand which controls IL-12 production at the tumor site. The protocol for rGBM trial allowed for patients to receive doses of veledimex between 10 and 40 mg daily over 14 days while patients with mBC received 80mg of veledimex daily for seven days.

In both tumor types, biopsy data showed consistent, dose-dependent production of IL-12 and interferon gamma and an influx of CD3⁺ CD8⁺ cytotoxic T cells. Additionally, there was evidence of sustained intratumoral increase in interferon gamma with undetectable levels of cytokines in systemic circulation in both studies. In the rGBM study, immunofluorescence studies show an overexpression of PD-1/PD-L1 markers.

In the mBC study, disease control rate was 44 percent at week 6 and 22 percent at week 12. Reductions in the diameter of both injected and non-injected lesions was observed and considered evidence of an abscopal effect.

Overall, Ad-RTS-hIL-12 plus veledimex revealed a consistent and attractive safety profile. In both studies, low grade dose-related transient cytokine release syndrome was the most commonly observed adverse reaction. Drug related toxicities were predictable, dose-related, and fully reversible upon discontinuation of veledimex.

About Controlled IL-12

Ad-RTS-hIL-12 plus veledimex is a novel gene therapy candidate designed to express human interleukin-12 (hIL-12) under the control of an orally administered activator ligand, veledimex, through a proprietary RheoSwitch Therapeutic System[®] (RTS[®]) gene switch. IL-12 is a powerful cytokine that has demonstrated a targeted, anti-tumor immune response with the ability to activate and recruit killer T cells to the tumor site. An ongoing Phase 1 trial is evaluating Ad-RTS-hIL-12 plus veledimex as a monotherapy to treat patients with rGBM, and a separate trial has been initiated to evaluate a single dose of Ad-RTS-hIL-12 plus veledimex in combination with OPDIVO[®] (nivolumab), an immune checkpoint inhibitor targeting programmed death receptor-1 (PD-1). The Company also is enrolling pediatric patients in its Phase 1 trial of Ad-RTS-hIL-12 with veledimex for the treatment of brain tumors at multiple U.S. sites. The Company also is exploring combination therapies with Controlled IL-12 and checkpoint inhibitors in additional tumor types.

About Ziopharm Oncology, Inc.

Ziopharm Oncology is a Boston-based biotechnology company focused on the development of next-generation immunotherapies utilizing gene- and cell-based therapies to treat patients with cancer. In partnership with Precigen Inc., a wholly-owned subsidiary of Intrexon Corporation (NYSE:XON), Ziopharm is focused on the development of two platform technologies designed to deliver safe, effective and scalable cell- and viral-based therapies for the treatment of multiple cancer types: Controlled IL-12 and *Sleeping Beauty* for genetically modifying T cells. The Company's lead asset, Ad-RTS-hIL-12 plus veledimex, has demonstrated in clinical trials the potential to control interleukin-12, leading to an infiltration of T cells that fight brain cancer. The Company also is advancing therapies using *Sleeping Beauty*, a non-viral approach to genetically modify chimeric antigen receptor (CAR⁺)

and T-cell receptor (TCR⁺) T cells, which target specific antigens in blood cancers and neoantigens in solid tumors. *Sleeping Beauty* is designed using the Company's point-of-care technology, a shortened manufacturing process which potentially can be developed as a decentralized manufacturing process based in hospitals. These programs are being advanced in collaboration with Precigen and with MD Anderson Cancer Center, the National Cancer Institute and Merck KGaA, Darmstadt, Germany.

Forward-Looking Disclaimer

This press release contains certain forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995, as amended. Forward-looking statements are statements that are not historical facts, and in some cases can be identified by terms such as "may," "will," "could," "expects," "plans," "anticipates," and "believes." These statements include, but are not limited to, statements regarding the Company's business and strategic plans as well as the progress and timing of the development of the Company's research and development programs, including the timing for the initiation of its clinical trials. All such statements are subject to certain risks and uncertainties, many of which are difficult to predict and generally beyond the control of the Company, that could cause actual results to differ materially from those expressed in, or implied by, the forward-looking statements. These risks and uncertainties include, but are not limited to: changes in the Company's financial condition and cash needs, funding or other strategic opportunities that become available to the Company, the Company's ability to finance its operations and business initiatives and obtain funding for such activities; whether chimeric antigen receptor T cell (CAR-T) approaches, Ad-RTS-hIL-12, TCR and NK cell-based therapies, or any of other product candidates will advance further in the preclinical research or clinical trial process and whether and when, if at all, they will receive final approval from the U.S. Food and Drug Administration or equivalent foreign regulatory agencies and for which indications; whether chimeric antigen receptor T cell (CAR-T) approaches, Ad-RTS-hIL-12, TCR and NK cell-based therapies, and the Company's other therapeutic products it develops will be successfully marketed if approved; the strength and enforceability of the Company's intellectual property rights; competition from other pharmaceutical and biotechnology companies; as well as other risk factors contained in the Company's periodic and interim reports filed from time to time with the Securities and Exchange Commission, including but not limited to, the risks and uncertainties set forth in the "Risk Factors" section of the Company's Annual Report on Form 10-K for the fiscal year ended December 31, 2017 and subsequent reports that the Company may file with the Securities and Exchange Commission. Readers are cautioned not to place undue reliance on these forward-looking statements that speak only as of the date hereof, and the Company does not undertake any obligation to revise and disseminate forward-looking statements to reflect events or circumstances after the date hereof, or to reflect the occurrence of or non-occurrence of any events.

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